
Statement from Dr. Maria Millan on the News of Orchard Returning the Treatment for ADA-SCID to UCLA

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Oakland, CA – The news that Orchard Therapeutics is returning OTL-101 to UCLA is wonderful news for families who have children with ADA-SCID. This means that UCLA will now be able to begin the process of applying to the Food and Drug Administration for permission to start an expanded use protocol to treat patients. This may take several months but CIRM will be helping and supporting UCLA at every stage of the process to make this happen as quickly as possible.

We know this has been a difficult time for the families of children with ADA-SCID and we are grateful to them for their patience and support as we worked through this. Throughout this process our sole purpose was to ensure that children in need were once again able to access this life-saving therapy. CIRM has supported the work of Dr. Don Kohn and his team for many years, and we are gratified to be able to help them once again.

We want to thank Dr. Bobby Gaspar and his team at Orchard and our colleagues at UCLA for working hard to make this happen. We have been in discussions for many months trying to find a way forward for this therapy, and now we have done that.

This is why CIRM was created. We are far more than just a funding agency. We are committed to doing everything we can to bring therapies to patients, and that means overcoming challenges and obstacles whatever they are. This is why we come to work every day, because patients are at the heart of everything we do.

This is a good day for CIRM, Orchard and UCLA. But it's a great day for our patients and their families.

Maria T. Millan, M.D., CIRM President & CEO

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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